Louis B Jacques, MD Responses to: Additional Questions for the Record from The Honorable Joseph R. Pitts – August 25, 2014

Q1. "In previous hearings…FDA approval is not the last hurdle…predictability of the CMS coverage process?"

It is important to acknowledge that some medical technologies are not intended for use in Medicare beneficiaries and thus CMS coverage policy is not determinative for them. In this context CMS may have no particular interest after FDA approval or clearance. However, CMS is appropriately expected to know what it pays for, and to have assurance that such items and services are reasonable and necessary as described in the statute. Unfortunately, some small and medium sized innovator companies seem focused on the requirements of FDA review and unmindful that Medicare and other payers have their own requirements for coverage and payment. Issues that could have been readily addressed well before FDA approval or clearance are therefore seen by some parties as last minute hurdles.

The most efficient way to address these issues for small and medium size companies is to support their earlier engagement with CMS and other payers regarding the questions that will arise in the context of coverage and payment. Both FDA and CMS have signaled interest in such a new paradigm, exemplified by the FDA-CMS Parallel Review pilot and other initiatives.

I believe the Medicare coverage process itself is predictable and transparent in comparison to some other Federal administrative processes and those of private companies. The challenge is that many small companies do not seem to be aware of the many published materials that describe and demonstrate examples of the coverage process and its outcomes. Similarly they seem unaware of the interest of CMS coverage staff to meet with innovators. In summary, they don't know what they don't know.

While the Parallel Review pilot is demonstrating positive results as recently as the August 11, 2014 proposed Medicare coverage of a DNA based screening test for colorectal cancer, the formal Parallel Review paradigm may be more than what is needed by some innovators. Given the geographic proximity of CMS and FDA offices, the establishment of a jointly supported interagency "innovator engagement space" could make these resources more obviously available and apparent to innovators. FDA could, as a standard procedure, inform these innovator companies of the availability of such voluntary early stage engagements.

Q2. "The most recent SGR patch legislation…"

Some members of the venture capital community have expressed interest in the establishment of a PAMA-like paradigm for truly innovative medical technologies, similar to provisions for advanced diagnostic tests. This could entail several PAMA-like components.

- A statutory definition that would define these technologies and distinguish them from incremental technologies. This might entail a combination of a) FDA PMA status; b) evidence of high likelihood of substantial new clinical benefit in a Medicare beneficiary population; and c) other factors established by the Secretary of HHS.

- Manufacturer product specific coding that would uniquely identify the item in the claims process.
- Payment calculation based initially on invoice amounts followed by a calculated payment rate that reflects commercial contracts.

Q3. "You state that CMS needs unambiguous authority to review clinical trials…"

Medicare serves beneficiary populations that are commonly excluded from enrollment in clinical studies because they have comorbid conditions associated with permanent disability, end stage kidney disease and advanced age that can complicate the interpretation of clinical trial results. In this context, the reported outcomes of some clinical trials do not describe how the studied technologies would impact these Medicare beneficiaries. Clearer incentives to enroll beneficiaries in clinical studies could support the development of better treatments for patients who have complicated chronic conditions, particularly the frail elderly patient.

As noted above in the response to the first question, some technologies are not intended for use in Medicare beneficiaries, and Medicare would not be asked to cover their clinical trials. However, CMS is appropriately expected to know what it does pays for, whether in clinical trials or usual clinical care. It is also reasonable to expect that Medicare coverage policy for research will reflect attention to the needs of the program and its beneficiaries. This will not happen if CMS has to rely on a piecemeal approach.

Current CMS authorities regarding coverage of items and services in clinical trials are siloed in three distinct vehicles: a) the June 2000 Executive Memorandum on coverage of routine clinical care costs in trials, i.e. usual care that the patient would get if not enrolled in a trial; b) statutory establishment of coverage of FDA approved IDE trials; and c) Coverage with Evidence Development (CED) under AHRQ's authority. This lack of integration stymies the development and publication of unified policy on the matter and unnecessarily lengthens the time to address coverage issues for clinical studies. Innovators seeking Medicare coverage for clinical trials don't have a unified set of criteria for reference. This engenders a reluctance to seek Medicare coverage for trials, with the consequence that innovators need to seek funding from other sources or conduct a more limited clinical study that may leave important questions unanswered.

The current paradigm does not address coverage in clinical studies for innovative technologies that may be studied in research sponsored by other Federal agencies or outside of an IDE or CED. Thus there may be missed opportunities to use the additional support that would be furnished by Medicare coverage to address important beneficiary-centric research questions.

Q4. "You state in your testimony that Local Coverage Determinations…"

Small device companies have historically expressed a preference to engage with local Medicare Administrative Contractors (MACs) as an alternative to opening a national dialog with CMS. As a practical matter, this approach represents a lower cost and lower risk strategy and may work well for a company whose facilities are entirely within a single MAC jurisdiction. However, the technology may be supported by preliminary evidence insufficient for coverage in the Medicare beneficiary population. A

broader LCD authority could permit earlier Medicare coverage on the condition of additional evidence development specifically to address the clinical impact of the device in the beneficiary population.

The current statutory definition of a Local Coverage Determination (LCD) is described in Section 1869(f)(2)(B) as:

> For purposes of this section, the term "local coverage determination" means a determination by a fiscal intermediary or a carrier under part A or part B, as applicable, respecting whether or not a particular item or service is covered on an intermediary–or carrier–wide basis under such parts, in accordance with section 1862(a)(1)(A).

This has the practical result of impeding the development of LCDs on any of the other "reasonable and necessary" provisions contained in Section 1862(a)(1)(A) of the Act. This also applies to clinical studies that might be supported under Coverage with Evidence Development, which is articulated by CMS as a decision under 1862(a)(1)(E) of the Act.